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PHARMNOTES

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Pharmacy Benefit Management
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Table of Contents

Drug Safety Alert Notification

- No drug safety communication published in December.

New FDA-Approved Drug Products

DRUG NAME	MANUFACTURER	APPROVAL DATE
FABHALTA™ (IPTACOPAN HYDROCHLORIDE) CAPSULES FOR ORAL USE	NOVARTIS PHARMACEUTICALS CORP.	12/5/2023

THERAPEUTIC CLASS
- Hematological Agents

FDA-APPROVED INDICATION(S)
- Fabhalta™ is a compliment factor B inhibitor, indicated for the treatment of adults with paroxysmal nocturnal hemoglobinuria (PHN).

DOSAGE AND ADMINISTRATION
- 200mg orally twice daily with or without food.

DOSAGE FORMS AND STRENGTHS
- Capsules: 200mg

Orphan status: Yes

SAFETY PROFILE

CONTRAINDICATIONS

- Serious hypersensitivity to iptacopan or any of the excipients.
- Initiation in patients with unresolved serious infection caused by encapsulated bacteria.

WARNINGS AND PRECAUTIONS
- **BLACK BOX WARNING: WARNING: SERIOUS INFECTIONS CAUSED BY ENCAPSULATED BACTERIA**
  - Fabhalta™ increases the risk of serious and life-threatening infections caused by encapsulated bacteria, including Streptococcus pneumoniae, Neisseria meningitidis, and Haemophilus influenzae type B.
- Complete or update vaccination for encapsulated bacteria at least 2 weeks prior to the first dose of Fabhalta™, unless the risks of delaying Fabhalta™ outweigh the risk of developing a serious infection. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for vaccinations against encapsulated bacteria in patients receiving a complement inhibitor.
- Patients receiving Fabhalta™ are at increased risk for invasive disease caused by encapsulated bacteria, even if they develop antibodies following vaccination. Monitor patients for early signs and symptoms of serious infections and evaluate immediately if infection is suspected.
  - Fabhalta™ is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called *Fabhalta™ REMS*.
- *Monitoring of PNH Manifestations After Fabhalta™ Discontinuation*: Monitor for signs of hemolysis after discontinuation.
- *Hyperlipidemia*: Monitor serum lipid parameters periodically during treatment and initiate cholesterol-lowering medication, if indicated.

ADVERSE REACTIONS
- Most common adverse reactions in adults with PNH (incidence ≥ 10%) were headache, nasopharyngitis, diarrhea, abdominal pain, bacterial infection, viral infection, nausea and rash.

USE IN SPECIFIC POPULATION
- *Severe renal impairment*: Use not recommended.
- *Severe hepatic impairment*: Use not recommended.
- *Pregnancy*: The use of Fabhalta™ in pregnant women or women planning to become pregnant may be considered following an assessment of the risks and benefits.
- *Lactation*: Since many medicinal products are secreted into human milk, and because of the potential for serious adverse reactions in a breastfed child, breastfeeding should be discontinued during treatment and for 5 days after the final dose.

DRUG NAME	MANUFACTURER	APPROVAL DATE
LYFGENIA™ (LOVOTIBEGLOGENE AUTOTEMCEL) SUSPENSION FOR INTRAVENOUS INFUSION	BLUEBIRD BIO, INC.	12/8/2023

THERAPEUTIC CLASS
- Hematopoietic Agents

FDA-APPROVED INDICATION(S)
- Lyfgenia™ is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of patients 12 years of age or older with sickle cell disease and a history of vaso-occlusive events.

DOSAGE AND ADMINISTRATION
- For autologous use only. For IV use only.
- Dosing is based on the number of CD34+ cells in the infusion bag(s) per kg of body weight.
- The minimum recommended dose is 3 × 10^6 CD34+ cells/kg.

DOSAGE FORMS AND STRENGTHS
- Cell suspension for IV infusion: a single dose of Lyfgenia™ contains a minimum of 3 x 10^6 CD34+ cells/kg of body weight, in one to four infusion bags.

Orphan status: Yes

SAFETY PROFILE

CONTRAINDICATIONS
- None

WARNINGS AND PRECAUTIONS
- **BLACK BOX WARNING: HEMATOLOGIC MALIGNANCY**
- Hematologic malignancy has occurred in patients treated with Lyfgenia™. Monitor patients closely for evidence of malignancy through complete blood counts at least every 6 months and through integration site analysis at Months 6, 12, and as warranted.
- *Delayed Platelet Engraftment*: Monitor patients frequently for thrombocytopenia and bleeding until platelet engraftment and platelet recovery are achieved.
- *Neutrophil Engraftment Failure*: Monitor absolute neutrophil counts (ANC) after infusion. If neutrophil engraftment does not occur, administer rescue cells.
- *Insertional Oncogenesis*: There is a potential risk of insertional
- oncogenesis after treatment.
- *Hypersensitivity Reactions*: Monitor for hypersensitivity reactions during infusion.

ADVERSE REACTIONS
- Most common adverse reactions ≥ Grade 3 (incidence ≥ 20%) were stomatitis, thrombocytopenia, neutropenia, febrile neutropenia, anemia, and leukopenia.

USE IN SPECIFIC POPULATION
- *Pregnancy*: There are no available data on Lyfgenia™ administration in pregnant women. Consider the risks associated with myeloablative conditioning agents on pregnancy and fertility.
- *Lactation*: Lyfgenia™ is not recommended for women who are breastfeeding, and breastfeeding after Lyfgenia™ infusion should be discussed with the treating physician.
- *Females and Males of Reproductive Potential*: Women of childbearing potential and men capable of fathering a child should use an effective method of contraception (intra-uterine device or combination of hormonal and barrier contraception) from start of mobilization through at least 6 months after administration of Lyfgenia™.
- *Pediatric*: The safety and efficacy of Lyfgenia™ in children less than 12 years of age have not been established.
- *Patients Seropositive for Human Immunodeficiency Virus (HIV):* A negative serology test for HIV is necessary prior to apheresis. Patients with a positive test for HIV will not be accepted for Lyfgenia™ treatment.

DRUG NAME	MANUFACTURER	APPROVAL DATE
CASGEVY™ (EXAGAMGLOGENE AUTOTEMCEL), SUSPENSION FOR INTRAVENOUS INFUSION	VERTEX PHARMACEUTICALS INCORPORATED	12/8/2023

THERAPEUTIC CLASS
- Hematopoietic Agents

FDA-APPROVED INDICATION(S)
- Casgevy™ is an autologous genome edited hematopoietic stem cell-based gene therapy indicated for the treatment of sickle cell disease (SCD) in patients 12 years and older with recurrent vaso-occlusive crises (VOCs).

DOSAGE AND ADMINISTRATION
- For autologous use only. For IV use only.
- Dosing of Casgevy™ is based on body weight. The minimum recommended dose is 3 × 10^6 CD34+ cells/kg .

DOSAGE FORMS AND STRENGTHS
- Cell suspension for IV infusion: The minimum recommended dose of Casgevy™ is 3 × 10^6 CD34+ cells per kg of body weight, which may be composed of multiple vials.

Orphan status: Yes

SAFETY PROFILE

CONTRAINDICATIONS
- None

WARNINGS AND PRECAUTIONS
- *Potential Neutrophil Engraftment Failure:* Monitor absolute neutrophil counts (ANC) after the infusion. Administer rescue cells in the event of neutrophil engraftment failure.
- *Prolonged Time to Platelet Engraftment*: Monitor platelet counts until platelet engraftment and recovery are achieved. Patients should be monitored for bleeding.
- *Hypersensitivity Reactions*: Monitor for hypersensitivity reactions during and after infusion.
- *Off-Target Genome Editing Risk*: Although not observed in healthy donors and patients, the risk of unintended, off-target editing in CD34+ cells due to uncommon genetic variants cannot be ruled out.

ADVERSE REACTIONS
- The most common Grade 3 or 4 non-laboratory adverse reactions (incidence ≥ 25%) were mucositis, febrile neutropenia, and decreased appetite.
- The most common Grade 3 or 4 laboratory abnormalities (≥ 50%) were neutropenia, thrombocytopenia, leukopenia, anemia, and lymphopenia

USE IN SPECIFIC POPULATION
- *Pregnancy*: Casgevy™ must not be administered during pregnancy because of the risks associated with myeloablative conditioning. Pregnancy after Casgevy™ infusion should be discussed with the treating physician.
- *Lactation*: The developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for Casgevy™ and any potential adverse effects on the breastfed child from Casgevy™ or from the underlying maternal condition. Breastfeeding after Casgevy™ infusion should be discussed with the treating physician.
- *Females and Males of Reproductive Potential*: Women of childbearing potential and men capable of fathering a child must use effective method of contraception from start of mobilization through at least 6 months after administration of Casgevy™.
- *Pediatric*: The safety and efficacy of Casgevy™ in children less than 12 years of age have not been established.
- *Patients Seropositive for Human Immunodeficiency Virus (HIV), Hepatitis B Virus (HBV) or Hepatitis C Virus (HCV):* Casgevy™ should not be used in patients with active HIV-1, HIV-2, HBV or HCV.
- *Patients with Prior HSC Transplant*: Casgevy™ has not been studied in patients who have received a prior allogeneic or autologous HSC transplant. Treatment with Casgevy™ is not recommended in these patients.

DRUG NAME	MANUFACTURER	APPROVAL DATE
IWILFIN™ (EFLORNITHINE) TABLETS FOR ORAL USE	USWM, LLC.	12/13/2023

THERAPEUTIC CLASS

- Antineoplastics and adjunctive therapies

FDA-APPROVED INDICATION(S)

- Iwilfin™ is an ornithine decarboxylase inhibitor indicated to reduce the risk of relapse in adult and pediatric patients with high-risk neuroblastoma (HRNB) who have demonstrated at least a partial response to prior multiagent, multimodality therapy including anti-GD2 immunotherapy.

DOSAGE AND ADMINISTRATION

- Prior to initiation of Iwilfin™, perform baseline audiogram, complete blood count, and liver function tests.
- Iwilfin™ is taken orally twice daily with or without food until disease progression, unacceptable toxicity, or for a maximum of two years
- Iwilfin™ tablets may be swallowed whole, chewed, or crushed and mixed with soft food or liquid.
- Recommended dosage of Iwilfin™ is based on body surface area

Body Surface Area (m^2)	Dosage
>1.5	768 mg (four tablets) orally twice a day
0.75 to 1.5	576 mg (three tablets) orally twice a day
0.5 to < 0.75	384 mg (two tablets) orally twice a day
0.25 to < 0.5	192 mg (one tablet) orally twice a day

DOSAGE FORMS AND STRENGTHS

- Tablets: 192 mg

SAFETY PROFILE

CONTRAINDICATIONS

- None

WARNINGS AND PRECAUTIONS

- *Myelosuppression*: Monitor blood counts before and during treatment with Iwlfin™. Withhold, reduce dose, or permanently discontinue based on severity.
- *Hepatotoxicity*: Monitor liver function tests before and during treatment with Iwilfin™. Withhold, reduce dose, or permanently discontinue based on severity.
- *Hearing Loss*: Monitor hearing before and during treatment with Iwilfin™. Withhold, reduce dose, or permanently discontinue based on severity.
- *Embryo-Fetal Toxicity*: Can cause fetal harm. Advise females of reproductive potential of the potential risk to a fetus and to use effective contraception.

ADVERSE REACTIONS

- Most common adversereactions (incidence ≥5%)are hearing loss, otitis media, pyrexia, pneumonia, and diarrhea.
- Most common Grade 3 or 4 laboratory abnormalities (incidence ≥2%) are increased ALT, increased AST, decreased neutrophil count, and decreased hemoglobin.

USE IN SPECIFIC POPULATION

- *Pregnancy:* Advise pregnant women and females of reproductive potential of the potential risk to a fetus.
- *Lactation:* Advise not to breastfeed
- *Contraception:* Advise females of reproductive potential to use effective contraception during treatment with Iwilfin™ and for 1 week after the last dose.

Orphan status: Yes

DRUG NAME	MANUFACTURER	APPROVAL DATE
FILSUVEZ™ (BIRCH TRITERPENES) TOPICAL GEL	LICHTENHELDT GMBH, PHARMAZEUTISCHE	12/18/2023

THERAPEUTIC CLASS
- Dermatological Agents

FDA-APPROVED INDICATION(S)
- Filsuvez™ topical gel is indicated for the treatment of wounds associated with dystrophic and junctional epidermolysis bullosa in adult and pediatric patients 6 months of age and older.

DOSAGE AND ADMINISTRATION
- Apply a 1 mm layer of Filsuvez™ to the affected wound surface and cover with wound dressing or apply Filsuvez™ directly to dressing so that the topical gel is in direct contact with the wound. Do not rub in the topical gel.
- Apply Filsuvez™ at wound dressing changes until the wound is healed.
- Each tube of Filsuvez™ is for one-time use only.
- For topical use; not for oral, intravaginal, intra-anal, or ophthalmic use.

DOSAGE FORMS AND STRENGTHS
- Topical gel: 10% birch triterpenes w/w supplied in 25 mL sterile tubes

SAFETY PROFILE

CONTRAINDICATIONS
- None

WARNINGS AND PRECAUTIONS
- *Hypersensitivity Reactions:* If signs or symptoms of hypersensitivity occur, discontinue use immediately and initiate appropriate therapy.

ADVERSE REACTIONS
- The most common (incidence ≥2%) adverse reactions are application site reactions.

USE IN SPECIFIC POPULATION
- *Pregnancy:* Systemic absorption of Fisulvez™ in humans is low following topical administration of Fisulvez™, and maternal use is not expected to result in fetal exposure to the drug.

Orphan status: Yes

DRUG NAME	MANUFACTURER	APPROVAL DATE
WAINUA™ (EPLONTERSEN) INJECTION, FOR SUBCUTANEOUS USE	ASTRAZENECA PHARMACEUTICALS LP.	12/21/2023

THERAPEUTIC CLASS

Psychotherapeutic and Neurological Agents

FDA-APPROVED INDICATION(S)

- Wainua™ is a transthyretin-directed antisense oligonucleotide indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults.

DOSAGE AND ADMINISTRATION

- The recommended dosage of Wainua™ is 45 mg administered by subcutaneous injection once monthly.
- Administer Wainua™ into the abdomen or upper thigh region; the back of the upper arm can be used if a healthcare provider or caregiver administers the injection.

DOSAGE FORMS AND STRENGTHS

- Injection: 45 mg/0.8 mL in a single-dose autoinjector.

SAFETY PROFILE

CONTRAINDICATIONS

- None

WARNINGS AND PRECAUTIONS

- *Reduced Serum Vitamin A Levels and Recommended Supplementation*: Supplement with the recommended daily allowance of vitamin A. Refer to an ophthalmologist if ocular symptoms suggestive of vitamin A deficiency occur.

ADVERSE REACTIONS

- Most common adverse reactions (that occurred in at least 9% of patients treated with Wainua™) were vitamin A decreased and vomiting.

Orphan status: Yes

New Biosimilar Products

Drug Name and Manufacturer	Date	Therapeutic Class	Indication(s)	Additional Information
Avzivi™ (bevacizumab-tnjn) injectable injection / Bio-Thera Solutions Ltd.	12/6/2023	Antineoplastics and adjunctive therapies	[1] Metastatic colorectal cancer, in combination with intravenous fluorouracil-based chemotherapy for first – or second- line treatment; [2] Metastatic colorectal cancer, in combination with fluoropyrimidine irinotecan- or fluoropyrimidine-oxaliplatin-based chemotherapy for second-line treatment in patients who have progressed on a first-line bevacizumab product-containing regimen; [3] Unresectable, locally advanced, recurrent or metastatic non-squamous non-small cell lung cancer, in combination with carboplatin and paclitaxel for first-line treatment; [4] Recurrent glioblastoma in adults; [5] Metastatic renal cell carcinoma in combination with interferon alfa; [6] Persistent, recurrent, or metastatic cervical cancer, in combination with paclitaxel and cisplatin, or paclitaxel and topotecan; [7] Epithelial ovarian, fallopian tube, or primary peritoneal cancer in combination with paclitaxel, pegylated liposomal doxorubicin, or topotecan for platinum-resistant recurrent disease who received no more than 2 prior chemotherapy regimens.	Avzivi™ is the second biosimilar researched, developed, and manufactured by a Chinese pharmaceutical company to receive FDA approval in the United States. Avzizi™ is the fifth biosimilar of Avastin™. Pricing and launch date are still pending. Reference Product: Avastin™ (bevacizumab) Orphan: No

New Formulations, Combination Products & Line Extensions

Drug Name and Manufacturer	Date	Therapeutic Class	Indication(s)	Additional Information
Phyrago™ (dasatinib) tablets, for oral use / Nanocopoeia, LLC	12/5/2023	Antineoplastics and Adjunctive Therapies	[1] Treatment of newly diagnosed adults with Philadelphia chromosome-positive (Ph+) chronic myeloid leukemia (CML) in chronic phase; [2] Treatment of adults with chronic, accelerated, or myeloid or lymphoid blast phase Ph+ CML with resistance or intolerance to prior therapy including imatinib; [3] Treatment of adults with Philadelphia chromosome-positive acute lymphoblastic leukemia (Ph+ ALL) with resistance or intolerance to prior therapy.	Orphan: Yes
iDose™ TR (travoprost intracameral implant) for intracameral administration) / Glaukos Corp.	12/13/2023	Ophthalmic Agents	Treatment indicated for the reduction of intraocular pressure (IOP) in patients with open-angle glaucoma (OAG) or ocular hypertension (OHT).	Orphan: No

New Formulations, Combination Products & Line Extensions

Drug Name and Manufacturer	Date	Therapeutic Class	Indication(s)	Additional Information
Zoryve™ (roflumilast) topical foam 0.3% / Arcutis Biotherapeutics, Inc.	12/15/2023	Dermatological Agents	Treatment of seborrheic dermatitis in adult and pediatric patients 9 years of age and older.	Orphan: No
Alyglo™ (immune globulin intravenous, human-stwk), 10% Liquid / GC Biopharma Corp.	12/15/2023	Passive Immunizing And Treatment Agents	Indicated for the treatment of primary humoral immunodeficiency (PI) in adults.	Orphan: No
Abacavir and Lamivudine tablets for suspension / Mylan Laboratories	12/22/2023	Antivirals	Treatment indicated in combination with other antiretroviral agents for the treatment of HIV-1 infection in pediatric patients aged 3 months and older and weighing at least 5 kg.	Orphan: No

New First-Time Generic Approvals

- No new first-time generics approved in December.

New FDA-Approved Indications for Existing Drugs

New FDA-Approved Indications

Drug Name and Manufacturer	Therapeutic Class	Previous Indication(s)	New Indication(s)	Date
Wilate™ (von Willebrand factor / Coagulation factor VIII complex (human)) injection / Octapharma	Hematological Agents – Misc.	[1] In children and adults with von Willebrand disease for: on-demand treatment and control of bleeding episodes, and perioperative management of bleeding. [2] In adolescents and adults with hemophilia A for: routine prophylaxis to reduce the frequency of bleeding episodes, and on-demand treatment and control of bleeding episode	Routine prophylaxis to reduce the frequency of bleeding episodes in children 6 years of age and older and adults with von Willebrand disease	12/1/2023
Jaypirca (pirtobrutinib) tablets, for oral use / Eli Lily and Company	Antineoplastics and Adjunctive Therapies	Adult patients with relapsed or refractory mantle cell lymphoma (MCL) after at least two lines of systemic therapy, including a BTK inhibitor.	Adult patients with chronic lymphocytic leukemia or small lymphocytic lymphoma (CLL/SLL) who have received at least two prior lines of therapy, including a BTK inhibitor and a BCL-2 inhibitor.	12/1/2023

New FDA-Approved Indications

Drug Name and Manufacturer	Therapeutic Class	Previous Indication(s)	New Indication(s)	Date
Welireg™ (belzutifan) tablets / Merck Sharpe Dohme	Antineoplastics and Adjunctive Therapies	For treatment of adult patients with von Hippel-Lindau (VHL) disease who require therapy for associated renal cell carcinoma (RCC), central nervous system (CNS) hemangioblastomas, or pancreatic neuroendocrine tumors (PNET), not requiring immediate surgery	For treatment of adult patients with advanced renal cell carcinoma following a programmed death receptor-1 or programmed death-ligand 1 inhibitor and a vascular endothelial growth factor tyrosine kinase inhibitor.	12/14/2023
Padcev™ (enfortumab vedotin-ejfv) lyophilized powder for injection / Astellas	Antineoplastics and Adjunctive Therapies	In combination with pembrolizumab for the treatment of adult patients with locally advanced or metastatic urothelial cancer who are not eligible for cisplatin containing chemotherapy	In combination with pembrolizumab for the treatment of adult patients with locally advanced or metastatic urothelial cancer	12/15/2023

Pipeline

Pipeline

Drug Name and Manufacturer	Date	Indication(s)	Additional Information	Impact
Arimoclomol / Zevra Therapeutics, Inc.	12/27/2023	For the treatment of Niemann-Pick disease Type C (NPC).	To address the issues raised in the CRL, the resubmitted NDA includes additional evidence to support the use of the NPCCSS and additional studies to support the potential mechanism of action. Moreover, the resubmission includes data from several nonclinical studies, natural history comparisons, real-world data from ongoing early access programs in the US and the European Union, along with data from the 4-year open-label extension of the phase 2/3 trial. NDA resubmitted.	High High

References

- *New Drug Approvals*. Drugs.com. (2023). https://www.drugs.com/newdrugs.html.
- *Latest Generic Drug Approvals*. Drugs.com. (2023). https://www.drugs.com/generic-approvals.html.
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- *New Drug Applications*. Drugs.com. (2023). https://www.drugs.com/new-drug-applications.html.
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